

## IND-enabling study of subretinal delivery of human neural progenitor cells for the treatment of retinitis pigmentosa

### Grant Award Details

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IND-enabling study of subretinal delivery of human neural progenitor cells for the treatment of retinitis pigmentosa

**Grant Type:** Late Stage Preclinical Projects

**Grant Number:** CLIN1-08235

**Project Objective:** The goal of the project is submission of an IND to support a phase 1/2a trial of hCNS-10-NPC in retinitis pigmentosa.

**Investigator:**

<b>Name:</b>	Shaomei Wang
<b>Institution:</b>	Cedars-Sinai Medical Center
<b>Type:</b>	PI

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**Disease Focus:** Vision Loss

**Human Stem Cell Use:** Adult Stem Cell

**Award Value:** \$4,954,514

**Status:** Active

### Progress Reports

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**Reporting Period:** Operational Milestone OM#1

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### Grant Application Details

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**Application Title:** IND-enabling study of subretinal delivery of human neural progenitor cells for the treatment of retinitis pigmentosa

**Public Abstract:****Therapeutic Candidate or Device**

human fetal cortex derived neural progenitor cells (CNS10-NPC)

**Indication**

To stabilize disease progression and maintain ocular integrity and vision for RP patients. This approach can be applied to more prevalent AMD.

**Therapeutic Mechanism**

A single subretinal injection of Human neural progenitor cells (CNS10-NPC) offers dramatic preservation of vision. CNS10-NPC engraft and migrate in the subretinal space, the desired site of action by photoreceptors. The CNS10-NPC secrete various growth factors and extracellular matrix (ECM) to stabilize the retinal health and preserve ocular integrity. In addition, grafted CNS10-NPC compensated the defective host RPE cells by phagocytizing photoreceptor outer segments. .

**Unmet Medical Need**

Retinitis pigmentosa (RP) constitutes a group of inherited disorders of progressive retinal degeneration affecting over 1.5 million people worldwide. There are diverse genetic causes of RP including up to 200 different mutations, there is no treatment available.

**Project Objective**

The goal of this project is to obtain an IND

**Major Proposed Activities**

- Obtain FDA regulatory approval to commence clinical trial with CNS10-NPS cells in RP subjects
- Complete dosing, toxicity and tumorigenicity and scale-up P23H pig studies sufficient to support IND for using CNS10-NPCs for treatment of RP
- Starting with the current Master Cell Bank, produce sufficient CNS10-NPCs for all pre-clinical and phase 1/2a clinical trial

**Statement of Benefit to California:**

Firstly, the project itself will employ new administrative, managerial, medical and scientific personnel in California. Secondly, the development of this stem cell based drug in California will ensure that the state retains its lead in the commercialization of stem cell technologies. Finally, if successful, this treatment would provide a substantial improvement to the vision of the 15,000 Californians with progressive blindness and other forms of progressive blindness including AMD.

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